

*Phase I/II Evaluation of Safety and Efficacy of a Matrix-targeted Retroviral Vector Bearing a Dominant Negative Cyclin G1 Construct (Mx-dnG1) as Adjunctive Intervention for Superficial Corneal Opacity/Corneal Scarring*

**Non-Technical Abstract**

The purpose of this study is to test the safety and potential efficacy of a gene transfer intervention in reducing the incidence of blurred vision (also known as "haze"), a known complication of laser eye treatment. The intervention consists of instillation of eye drops containing a genetically modified virus that serves as a vehicle (vector) carrying a modified human gene (dnG1) that inhibits the multiplication of certain cells in the eye that cause the "haze" as an abnormal wound healing response to laser treatment. In animal studies, a targeted vector that was further modified to seek out injured tissues (known as Mx-dnG1) was more effective than the non-targeted vector bearing the same gene in reducing the occurrence of "haze" after laser surgery in rabbits' eyes.

The clinical protocol will include 9 to 15 eligible persons who are scheduled for laser eye treatment for scarring of the cornea (the outer covering of the lens of the eye). The participant will be assigned to receive a specific dose according to the approved clinical protocol. Each dose will be administered as eyedrops every 30 minutes to 1 hour for ~12 hours for three consecutive days after surgery. Three increasing dose levels will be given. Patients will be evaluated at specified intervals for a period of one year following the gene transfer intervention. Possible benefits of the study are prevention or reduction of blurred vision or "haze", which could obviate the need for more invasive procedures such as corneal transplantation. The risks of this intervention include possible occurrence of corneal ulceration, poor wound healing, development of antibodies against the vector, and a very small risk of causing cancer, although this has not been reported in anyone who has received this type of intervention. Other risks are essentially those related to the laser surgery which includes worsening of vision, corneal ulceration or perforation of the cornea, and infection.